

~~CB-249,689~~

DESIGNING COMPOUNDS SPECIFICALLY INHIBITING RIBONUCLEIC ACID

Abstract of the Invention

A method for designing compounds specifically targeting RNA sequences, based on the discovery of short, specific sequences within RNA that are critical to function, using modeling of the compound to effect binding to the nucleotide sequences in the RNA in combination with secondary and/or tertiary structure associated with the minor groove of the RNA in the region of the critical sequences. In the preferred method, computer modeling is used along with analysis of the targeted RNA sequence to design molecules binding to the targeted RNA by covalent or hydrogen binding. Appropriate molecules are synthesized using known methodology that have the required structure and chemical characteristics to specifically bind the critical region of the RNA and thereby inhibit the function of the RNA. Molecules known to bind to RNA can also be modified using this method to increase specificity, and thereby decrease toxicity.